



Make today a breakthrough.

January 12, 2021

Dear Member of Congress,

As you prepare for the 117th Congress, Cure SMA is pleased to **share background on spinal muscular atrophy**, a neuromuscular disease, and to highlight **legislative issues important to children and adults with SMA and their families**. We respectfully ask that you and your legislative team consider and advocate for the priorities and concerns of the SMA community during the upcoming legislative session.

Spinal muscular atrophy (or SMA) is a **progressive neurodegenerative disease that robs individuals of their physical strength**, impeding their ability to walk, swallow, and breathe. SMA affects approximately 1 in 11,000 births, and about 1 in every 50 Americans is a genetic carrier. If both parents are SMA carriers, every child they have together has a 25% chance of being diagnosed with SMA, regardless of race and ethnicity.

Recent developments in treatment, advances in care, and progress toward universal newborn screening provide a bright future for the SMA community. There are now three effective U.S. Food and Drug Administration-approved treatments for SMA that make it possible for individuals with SMA to achieve unprecedented milestones and stop or slow the degenerative course of the disease. In addition, another 17 states started to screen for SMA in 2020, bringing the total to 33 SMA newborn screening states. By the end of 2021, Cure SMA projects about 9 in every 10 babies born in the U.S. will be screened for SMA, once considered the number one genetic cause of death for infants. Newborn screening gives parents of babies born with SMA the information they need to make timely decisions about treatment and care.

Together, these accomplishments mean that children and adults with SMA are thriving in and fully contributing to their communities. Individuals with SMA may utilize care, services, and equipment available through Medicaid and other government-funded programs to increase their independence and to assist with activities of daily living. In addition, individuals with SMA benefit from the protections and rights available through federal law. As such, decisions made by the 117th Congress could significantly impact individuals with SMA and their families.

Cure SMA is the leading national organization that represents individuals with SMA and their families from your state and across the country. From funding basic research into SMA treatments and developing an evidence-based standard of care to delivering equipment and support programs and hosting events and educational forums, Cure SMA is the indispensable resource and advocate for the SMA community. Throughout the 117th Congress, Cure SMA will strongly advocate to ensure that individuals with SMA have full access to all aspects of life, from education and employment to health care, transportation, and housing.

Cure SMA's legislative priorities for the 117th Congress include:

- **EDUCATION:** Access to high-quality, inclusive education and educational supports—from early education to higher education—are critical for individuals with SMA.



- **EMERGENCY MANAGEMENT:** Effective emergency preparedness and disaster response are a matter of life and death for individuals with SMA and other disabilities.
- **EMPLOYMENT & FINANCIAL SECURITY:** Adults with SMA identified flexible work schedule and financial independence as two of their top unmet needs, according to a recent Cure SMA survey.
- **HEALTH CARE:** Full and timely access to health care, treatments, and health-related supports greatly increase the health, well-being, and independence of individuals with SMA.
- **HOUSING:** Full community integration and participation for individuals living with SMA and other disabilities require full and equal access to affordable and physically accessible housing.
- **NEWBORN SCREENING:** Diagnosis through newborn screening and early treatment can lead to improved, long-lasting developmental outcomes for individuals with SMA.
- **RIGHTS:** Individuals with SMA should not be discriminated against and should have equal opportunity, full participation, independent living, and economic self-sufficiency, as mandated by federal law.
- **SMA RESEARCH:** Research into treatment, care, and equipment promotes independence and healthy living for individuals with SMA.
- **TECHNOLOGY:** Assistive technology devices and other technological advances can assist individuals with SMA in education, employment, and other activities of daily living.
- **TRANSPORTATION:** Transportation remains a top barrier for individuals with SMA, which impacts their ability to access healthcare, education, housing, employment, and community services.

Thank you for considering the priorities and views of individuals with SMA and their families.

Please do not hesitate to contact Cure SMA to answer questions or to provide feedback to your office on how legislative proposals may impact individuals with SMA living in your state. Your staff can contact Maynard Friesz, Cure SMA's Vice President for Policy and Advocacy, at maynard.friesz@curesma.org or 202-871-8004.

Sincerely,

Kenneth Hobby
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Cure SMA

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